

Ensuring the safety of cell therapy: a quality control pipeline for cell purification and validation

Grant Award Details

Ensuring the safety of cell therapy: a quality control pipeline for cell purification and validation

Grant Type: Early Translational I

Grant Number: TR1-01250

Project Objective: Goal = address the safety bottleneck of teratoma and off target tissue formation risk from pluripotent stem cell (PSC) derived cell therapies via:
Development of very sensitive assays, residual PSC purging, off target differentiation purging.

Investigator:

Name: Jeanne Loring
Institution: Scripps Research Institute
Type: PI

Name: Andrew Laslett
Institution: Chapman University
Type: Partner-PI

Collaborative Funder: Victoria, Australia

Human Stem Cell Use: Embryonic Stem Cell, iPS Cell

Award Value: \$5,830,771

Status: Closed

Progress Reports

Reporting Period: Year 1

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Reporting Period: Year 2

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Reporting Period: Year 3

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Reporting Period: NCE

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Grant Application Details

Application Title: Ensuring the safety of cell therapy: a quality control pipeline for cell purification and validation

Public Abstract: The clinical application of cell replacement therapy in the US is dependent on the FDA's approval, and the primary objective of the FDA is to protect patients from unsafe drugs and procedures. The FDA has a specific mandate for human gene and cell therapy and since the unexpected deaths in early trials of gene therapy trials the bar for safety in these areas is unusually high.

This is a summary of the key findings from the FDA's report on human embryonic stem cell therapy (April 2008): "From the perspective of toxicology, the proliferative potential of undifferentiated human embryonic and embryonic germ cells evokes the greatest level of concern. A characteristic of hESCs is their capacity to generate teratomas when transplanted into immunologically incompetent strains of mice. Undifferentiated hESCs are not considered as suitable for transplantation due to the risk of unregulated growth. Before clinical trials are begun in humans, the issue of unregulated growth potential and its relationship to stem cell differentiation must be evaluated".

In order to overcome the concerns about the safety of pluripotent stem cell therapy, we have designed a pipeline of quality control measures that can be applied to any cell type that is being considered for Investigational New Drug (IND) approval by the FDA. The technologies that we will develop under this award will allow rigorous selection and characterization of cells before they are tested as transplants. By reducing the possibility that stem cell therapies will be toxic or cause cancer in patients, we will remove the major barriers to advancement of these therapies to the clinic.

**Statement of Benefit to
California:**

Californians are a large and diverse population that poses unique challenges for the future of medical care. Fortunately, California has a tradition of taking the lead in technology and medical breakthroughs and following through from the first idea to the final product. Almost 20,000 Californians await organ transplants, and more than a million suffer from progressive degenerative diseases and injuries such as Alzheimer disease, Parkinson's disease, nerve-muscle disease such as amyotrophic lateral sclerosis (ALS) and muscular dystrophy, liver disease, diabetes, and spinal cord injury. The possibility of applying cell replacement therapy to these problems could drastically improve the outlook for treatment for the victims. A major goal for California's supporters of stem cell research is development of stem cell-based products that have medical use, and the mandate for the research community is to provide the best possible fundamental information to help guide clinical applications to make these cells as safe as is possible for cell therapy, by ensuring that they retain normal, noncancerous qualities.

California scientists have taken the first steps to clinical applications of pluripotent stem cells through their cutting edge research in developing new ways to derive these cells and to differentiate them into cell types that can be used to replace damaged tissues. We propose to take this research to the next step, to prepare the cells for clinical trials. We propose to develop a comprehensive pipeline of quality control technologies that will ensure the safety and purity of cells used first for preclinical testing and later for obtaining IND approval from the FDA for initiating human trials. These technologies can be used for any cell therapy, and will considerably reduce the barriers to development of safe, effective new treatments for incurable disease. This will have a positive effect on the health care of all Californians, reduce the cost of development of cell therapies, and create new opportunities for jobs and industry in the state.

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